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RNA Editing for Muscular Dystrophy Therapy

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Abstract

© 2016, Springer Science+Business Media New York.Due to lack of effective therapies, muscular dystrophies became a focus for gene therapy. Multiple pre-clinical studies have shown successful restoration of dystrofin and dysferlin by RNA editing both in vivo and in vitro, but possibility of a clinical translation is still obscure. A number of new chemicals are being studied, and a search for new techniques is ongoing. This work is intended to give a brief overview of the current state of the RNA editing for treating muscular dystrophies.

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Keywords

Duchenne muscular dystrophy, Dysferlinopathy, Exon skipping, RNA editing, Trans-splicing

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